



## FREQUENTLY ASKED QUESTIONS

**1. What does WBDR stand for?**

WBDR stands for the World Bleeding Disorders Registry

**2. What is the WBDR?**

The WBDR is an online web-based data entry system that provides a platform for a network of hemophilia treatment centres (HTCs) around the world to collect uniform and standardized patient data and guide clinical practice.

With informed consent from the patient, the WBDR stores anonymous data about the person's disease, such as hemophilia type and severity, symptoms and treatment. This de-identified and confidential data will be catalogued and stored with the aim of sharing the data with the scientific community, helping them to address important questions around patient care and advocacy/health policy issue.

**3. Why do we need the WBDR?**

Despite significant progress in the development of safe and efficacious hemophilia treatments over the past 50 years, there are still considerable differences in treatment practices, access to therapies and patient outcomes in much of the world. The WBDR is intended to fill these gaps in care and knowledge by collecting real world data on the patient clinical experience around the globe. Researchers and policy influencers will be able to use this patient data to generate evidence and build advocacy initiatives aimed at health policy decision makers.

The WFH is proud to partner with HTCs, national member organizations, and people around the world living with hemophilia, as each plays an active role in building this important resource and improving the future of hemophilia care.

**4. How does the WBDR work?**

Healthcare providers from participating HTCs will talk to each of their hemophilia patients about being enrolled into the WBDR. HTCs will enroll patients who consent to have their details included in the registry. This database will then be available to the global scientific community.



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### 5. What are the goals of the WBDR?

The WBDR aims to improve the quality of care for people living with hemophilia around the world. Following the success of the WBDR pilot study, the WBDR is now in full-scale global implementation. The goals of the WBDR are for at least 200 HTC's from more than 50 countries to participate, and for at least 10,000 people with hemophilia to be enrolled during the first five years.

### 6. Why is the WBDR important?

Registries, with international collaboration between centres and countries, are an effective way to pool data to achieve the required sample size to conduct clinical research on rare disorders, such as hemophilia.

By combining data from countries around the world, the WBDR will provide a large amount of data, on which researchers can address important scientific and clinical issues.

### 7. How are HTC's selected and invited to participate in the WBDR?

The WFH aims to have patients from around the world and from all levels of access to care represented in the WBDR.

HTC participation criteria are minimal and include:

- Change to internet access to connect with the web-based data entry system.
- Personnel resources available to input data in the registry.
- Commitment to long-term enrolment practices and comprehensive patient follow-up.



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**8. Who can participate in the WBDR?**

Patients diagnosed with hemophilia A or B who are registered at one of the participating HTC's are eligible for participation in the WBDR.

**9. How will patients benefit from participating in the WBDR?**

By providing information about their experience with hemophilia, people with hemophilia play an active role in helping researchers better understand hemophilia and improve the quality of care and treatment for people with hemophilia around the world.

Collecting uniform and standardized data on the clinical care people with hemophilia are receiving around the world will allow researchers to compare country specific levels of care, and use this data to advocate for better care. People with hemophilia who participate in the registry will also have the opportunity to ask research questions to the WBDR Research Steering Committee, once data starts to accumulate in the database.

**10. How will HTC's benefit from participating in the WBDR?**

HTC's will be able to use the WBDR database as a means of tracking patients and monitoring patient outcomes, as well as guiding clinical practice. Participating in the WBDR will allow HTC's to answer important scientific and clinical questions using data from the global database.

**11. What about the privacy of participants?**

All of the disease information that is entered in the WBDR is anonymous and confidential. Patient names or other identifying information will not be entered into the database. Only WBDR project team will be able to view the anonymized data.



## FREQUENTLY ASKED QUESTIONS

### **12. What about the protection of the data?**

The WBDR database is being developed through a collaboration between the WFH, Karolinska Institute based in Sweden and Health Solutions, also based in Sweden.

Data policy guidelines of Health Solutions adhere to both the CE-mark (Conformité Européenne) and the UK standard IGSoC (Information Governance Standard of Compliance).

The WBDR database will be compliant with the new General Data Protection Regulation which will be enforced in the European Union as of 25 May 2018.

### **13. Has there been a pilot of the WBDR?**

Yes, a successful pilot study was completed in 2016, with 26 HTC's participating and 356 patients enrolled. The approval of the full scale WBDR was based on the success of the pilot study.

### **14. What lessons were learned from the pilot that will be considered going forward?**

Feedback from HTC pilot participants were very positive overall, as assessed by a post-pilot questionnaire. The pilot demonstrated great interest and ability from HTC's, ethics committees and patients worldwide to successfully participate in a web-based patient registry.

The main issues identified by HTC's during the pilot study included obtaining ethics approval, limited staff resources, and technical difficulties. The WFH addressed these challenges by allowing additional time for ethics review and resourcing, and resolving the technical issues. WFH will continue to build upon the pilot learnings throughout the full-scale launch of the program.

### **15. How is the WBDR funded?**

WFH is an international not-for-profit organization with recognition from the World Health Organization. It receives funding through donations, government grants and corporate sponsorship which will help support the WBDR. Initial funding has been confirmed for the launch of the full scale WBDR.